
2011 Annual Report: Smoothing the path to therapies

News at CIRM

Smoothing the Path to Therapies

Fostering partnerships between academic researchers and industry might not be considered a traditional role for a funding agency, but in today's changing financial environment, making connections between stem cell scientists and industry is critical if the research CIRM funds is going to result in new therapies. That's why in the past year CIRM has ramped up its efforts to help grantees with the critical skills and information they'll need if their work is to reach clinical trials in patients.

Changing landscape

Researchers today are carrying out much of the work that was once largely the domain of industry. Up until fairly recently companies and venture capitalists were the primary source of funding for the early stages of research into possible therapies. But when a venture capital firm makes an investment it wants to see it pay off within a short time frame. Because new drugs can cost close to \$4 billion to develop and take 10 to 15 years to get to market there is a lot at stake. As a result there is now a trend for investors to hold back before diving in, to wait for evidence that the potential therapy is safe and is effective in animals, or even until after the earliest phases of human trials.

That change leaves many investigators on their own in those early stages of therapy development. To carry their research through to the point where it is an attractive investment for industry partners means learning to navigate the many challenges of understanding manufacturing and regulations surrounding new therapy development—skills that investigators don't typically learn in graduate school.

Barriers to investment

Today researchers often need to have thought about issues such as how their prospective therapy will be manufactured and how much it will cost because a therapy candidate that costs less to produce will naturally be a more attractive investment.

The same holds for understanding how the proposed therapy will be regulated by the Food and Drug Administration, which makes decisions about which therapy candidates can be tested in clinical trials. A clearer regulatory path is also a faster path to the market.

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"One thing is that you have to have your target very clear and you have to know what regulations apply because those can effect your timeline," said Mahendra Rao, who directs the National Institutes of Health Intramural Center for Regenerative Medicine. A shorter timeline means a faster pay-off, which is attractive to investors. "That's why CIRM needs to involve the late stage folks early. They need to encourage researchers to include pharma now, to say 'I am starting my screening, what should I put into it?'"

Stem cell-based therapies face additional hurdles in reaching patients because the field is so new. So far, just three trials with embryonic stem cells have made it through FDA regulations and into clinical trials.

Duane Roth, who is co-vice chair of CIRM's governing board, said that the three Rs of therapy development—Research, Regulatory pathway, and Reimbursement—are all barriers for stem cell therapies to overcome in the established path to the market. Roth is Chief Executive Officer of CONNECT, a non-profit organization that fosters entrepreneurship in the San Diego area.

"There are so many things in the research phase that are ill defined, and the regulatory process is ill defined," Roth said. "The numbers don't add up. How does the company get paid to earn a profit?"

What we're doing

To help investigators navigate their way through all these challenges CIRM has launched a series of initiatives that are targeting partnerships with the biotech, venture capital and pharmaceutical industries. These are broadly under the umbrella of the Opportunity Fund that tailors CIRM loans and grants to better fit the needs of industry. CIRM's President Alan Trounson believes "the calls for

applications under these umbrellas will result in very significant co-funding of stem cell companies and their early clinical trials, and the relocation of major companies to California to join the rapid moving front line of high quality medical applications that are evolving from the CIRM translational pipeline".

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These initiatives have been aimed at both connecting investigators with industry partners and also engaging with the FDA to help clarify the regulatory path for stem cell therapies. CIRM has offered free webinars that included members from industry and the FDA talking about issues that face products on the path to clinical trials. Those remain available on the agency's website for researchers seeking information. (CIRM's webinars are indexed [here](#).)

This November CIRM also co-hosted an investor partnering program event to bring stem cell scientists, investors and industry together. Several of CIRM's first round disease teams presented their projects to an audience of possible future investors. Those researchers had a chance to attend sessions on the regulatory pathway, accessing finance and developing business models—all topics that today's scientists need to understand if their projects are going to continue into the clinic.

A \$30 million funding initiative committed this year will expand these existing efforts to promote academic partnerships with industry. Elona Baum, CIRM General Counsel and Vice President of Business Development, led the effort to develop this fund.

"We want to increase the likelihood that CIRM research has access to funding in the later stage of development which is critical to ensuring it gets into the market place and helps patients in need," Baum said. "We're being proactive by engaging industry early in the process of developing new therapies." (Read the press release about those funds.)

Bring in the Advocates

Roth acknowledges that getting academic scientists and industry talking is a good first step to speeding therapies, but thinks there's one group missing: patient advocates.

Serving on CIRM's governing board with ten patient advocates changed Roth's perception about the role of the advocate in promoting new therapies. "They have an incredible ability to focus on the benefit because it's personal," he said. (CIRM's governing board members are listed [here](#).)

Because the safety of a possible therapy rests with the FDA, the organization tends to be risk-adverse. He advocates having the people who would be taking the risk in the room. "Imagine if you had a third opinion," Roth said. "I promise you that's a report people will listen to."

In today's investment climate, getting therapies to market requires more than just funding research—the traditional role for a funding agency. CIRM's efforts to connect academic researchers with industry, involve patient advocates earlier, and engage with the FDA means more of what we fund will eventually reach the people who need them most—the patients.

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